2019 PDA Cell and Gene Therapy Conference

Concept through Commercialization – Patient-Focused Manufacturing

May 6-7, 2019 | Hilton Long Beach | Long Beach, CA

MONDAY, MAY 6

7:00 a.m. – 8:30 a.m. | Continental Breakfast

8:30 a.m. – 10:30 a.m. | P1: The Patient Perspective: How Innovative Therapies are Transforming Lives

Innovative cell and gene therapies have drastically changed how we address the technical challenges in developing and manufacturing these medicines. These products have also drastically changed the patient experience by providing a new set of options for the patient that were, not very long ago, simply pipe dreams. This session will explore how these innovative therapies impact patients' lives and therefore help provide the audience with the necessary context as we follow the path toward commercialization.

Moderator: Michael Blackton, MBA, Vice President, Global Quality, *Adaptimmune, LLC*

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8:30 a.m. | Welcome and Opening Remarks from Conference Co-Chair
Michael Blackton, MBA, Vice President, Global Quality, *Adaptimmune, LLC*

8:45 a.m. | Journey to T-Cell Therapy, The Whitehead Family
Tom G. Whitehead, Co-Founder, *Emily Whitehead Foundation*

9:30 a.m. | Supplying Cell and Gene Therapies: It's Going to Take More Than Cutting-Edge Science Alone for us to Serve our Patients
Thomas A. Leitch, Vice President, Vector Manufacturing, *bluebird bio*

10:00 a.m. | Questions and Answers/Discussion

10:30 a.m. – 11:00 a.m. | Refreshment Break and Poster Presentations

Poster Presentations

The following posters will be presented during refreshment breaks on Monday and Tuesday

1. **Analytical Characterization of a Complex Product: Lentiviral Vectors**
   Julia Deuel, MS, Senior Scientist, *bluebird bio*
2. **Expand Outside the Hood: Cell Passaging Outside Biosafety Cabinet with Fully Closed and Aseptic in Cell Expansion System**
   Charles P. Meadows, MBA, Product Manager, *Sartorius Stedim Biotech*
3. **Electronic Solutions for Manufacturing Challenges**
   Crystal M. Booth, Regional Manager, *PSC Biotech*
4. **Particulate Matter in Single-Use Systems: Measurement and Risk Reduction**
   Klaus R. Wormuth, PhD, Lead Scientist, *Sartorius Stedim Biotech*
5. **Validation of a Fully Automated Mycoplasma Detection Method Intended to Improve the Ease-of-Use and Time-to-Result for Nucleic Acid Tests**
   William Barry, PhD, Scientist, *BioFire Defense*
6. **Leveraging High-Dimensional '-Omics' Technologies for Comprehensive Profiling of CAR T Cells to Resolve Drug Product Complexity**
   Eric S. Alonzo, PhD, Scientist, *bluebird bio*
7. **Advanced LC-MS Approach for Characterization of HCPs and Delivery Vehicle (Viral) Proteins in Gene Therapy Products**
   Dongdong Wang, PhD, Director, *BioAnalytix*

11:00 a.m. – 12:30 p.m.| P2: The A, B, C's of Cell and Gene Therapy Facility Design

The facility design for cell and gene therapy products must take into consideration the many aspects of the manufacturing process. Most processes involve manual aseptic manipulations performed within a biological safety cabinet and/or a combination of manual aseptic manipulations and automation. Additionally, the facility design must incorporate flexibility in the manufacturing areas as well as the need for future expansion. This session will explore approaches to consider when designing and/or modifying a facility to ensure a compliant, yet seamless end-to-end manufacturing process for cell and gene therapy products during evolving and non-harmonized regulatory landscapes.

Moderator: Irving Ford, MSc, Head, CAR-T QC Laboratories, *Celgene, Biotechnology Company*

11:00 a.m. | Flexible Gene Therapy Facilities: Current Best Practices
John E. Dougherty, Lead Process Engineer, *DPS Group*

11:30 a.m. | CGMP Considerations for the Design and Operation of Cell Therapy Facilities
Francesca A. McBride, Director Regulatory Compliance, *Jacobs*

12:00 p.m. | Questions and Answers/Discussion

12:30 p.m. – 1:30 p.m. | Networking Lunch

MONDAY, MAY 6, CONTINUED

1:30 p.m. – 3:00 p.m. | P3: Tech Transfer and Comparability for Cell and Gene Therapies: Case Studies in Complexity

Accelerated clinical development timelines and regulatory pathways, highly complex products, and a still-maturing manufacturing infrastructure can lead to significant challenges for cell and gene therapy CMC organizations. Multiple site transfers, limited batch sizes, and high inherent process variability are just a few of the hurdles to be cleared on the sprint from first-in-human to commercial licensure. We will explore case studies that reveal strategies for technology transfer and comparability that help to navigate these unique challenges while maintaining the speed and flexibility that is critical to this rapidly maturing field.

Moderator: Michael Kuczewski, Associate Director, Oncology CMC, *bluebird bio*

1:30 p.m. | Launching Clinical Trials in Europe: A QP's Perspective
Stephanie M. Verbrugghe, QP, CPGP, CEO & Founder, *Farbridge Pharma Consulting, LLC*

2:00 p.m. | Regulatory Perspective on Tech Transfer and Comparability
Steven S. Oh, PhD, Deputy Director, Division of Cellular and Gene Therapies, CBER, *FDA*

2:30 p.m. | Questions and Answers/Discussion

3:00 p.m. – 3:30 p.m. | Refreshment Break and Poster Presentations

3:30 p.m. – 5:00 p.m. | P4: Navigating the Product Characterization Process

Cell and gene therapies present unique challenges related to product characterization and potency testing relative to traditional biologics. The link between product attributes/potency, starting material, methods, and clinical relevance can be poorly understood or may have little predictive value in many cases, while regulatory expectations are ambiguous. These case studies will explore recent challenges faced by companies during product characterization and development of potency methods.

Moderator: Eden S. Fucci, Vice President of Biologics Manufacturing, *Torque Therapeutics*

3:30 p.m. | Phase Appropriate Lifecycle Management of Analytical Methods to Support Gene Therapy Products
Brendan G. Keenan, MS, PhD, Associate Director Quality Control Sciences and Technology, *bluebird bio*

4:00 p.m. | Enhancing Our Product Knowledge and Understanding of Cell Therapy Products through Comprehensive Analytical Characterization of Patient Material
Tam Soden, PhD, Senior Director and Head of Analytical Development, *Kite, a Gilead Company*

4:30 p.m. | Questions and Answers/Discussion

5:00 p.m. – 6:00 p.m. | Networking Reception in Exhibit Area

TUESDAY, MAY 7

7:00 a.m. – 8:45 a.m. | Continental Breakfast

7:15 a.m. – 8:30 a.m. | Cell and Gene Therapy Interest Group Breakfast Session

One of the key challenges in cell and gene therapies is the need to thoroughly understand advanced products and processes. Analytics provide the key to understanding critical attributes of advanced products and processes as they advance toward commercialization. Given this, robust strategies for the development, qualification, and validation of innovative analytical techniques is an absolute requirement for success. In this session we will explore novel approaches to incorporation of analytical strategies supporting product and process understanding.

Moderator: Michael Blackton, MBA, Vice President, Global Quality, *Adaptimmune, LLC*

7:15 a.m. | Replication Competent Retrovirus Testing for Gene Therapy Vectors: Current Methodology and Regulatory Expectations
Leyla S. Diaz, PhD, Principal Scientist, *MilliporeSigma*

7:45 a.m. | Potency Assay Development and Qualification for B-Thalassemia and Sickle Cell Disease Autologous Gene Therapy Drug Products
Ilya A. Shestopalov, PhD, Associate Director, Cell Analytics, *bluebird bio*

8:15 a.m. | Questions and Answers/Discussion

8:45 a.m. – 10:15 a.m. | P5: Application of Quality Risk Management Principals in Cell and Gene Therapy

The use of quality risk management (QRM) principals and risk-based approaches are a regulatory expectation. This concept is highlighted in the draft revision of Annex 1. Risk-based approaches are needed in all aspects of cell and gene therapy low bioburden and sterile manufacturing. This session will review specific case studies of implementation of risk-based approaches and QRM principals in the design, control, monitoring, and operation of cell and gene therapy processes.

Moderator: Brian J. Hawkins, PhD, Chief Technology Officer, *Pluristyx, Inc.*

8:45 a.m. | Executing a Successful Microbial HACCP: Avoid Doing a Check the Box Risk Assessment and Calling it Done
Marsha Steed, Director Global QC Microbiology and Contamination Control, *bluebird bio*

9:15 a.m. | Using the Intervention Risk Evaluation Model (IREM) to Build a Risk-Based Approach to Operator Qualification and APS
Dr. Mike Long, MBB, Senior Director Consulting Services, *ValSource LLC*

9:45 a.m. | Questions and Answers/Discussion

10:15 a.m. – 11:00 a.m. | Refreshment Break and Poster Presentations in Exhibit Area

11:00 a.m. – 12:30 p.m. | P6: Raw Material Selection and Control for Cell and Gene Therapy Manufacturing

Raw material selection and control presents unique challenges in cell and gene therapy manufacturing over traditional biopharmaceutical manufacturing. In this session we will explore some of these challenges and offer practical approaches for managing these hurdles using risk assessments and robust control strategies.

Moderator: Kimberly A. Carnes, Director, Quality Systems, *REGENXBIO*

11:00 a.m. | The Role of Virus Filtration in Achieving Pathogen Safety of Cell and Gene Therapy Products
Sebastian B. Teitz, PhD, Scientific Coordinator, *ASAHI Kasei Bioprocess*

11:30 a.m. | Building an Agile Raw Material Supply Chain for Gene Therapy Manufacturing
Stefanie E. Brady, Director of Supply Chain, *REGENXBIO*

12:00 p.m. | Questions and Answers/Discussion

12:30 p.m. – 1:30 p.m. | Networking Lunch

TUESDAY, MAY 7, CONTINUED

1:30 p.m. – 3:00 p.m. | P7: Needle to Needle Visibility and Supply Chain Challenges

Cell therapy manufacturing begins with a collection of cells or tissue from the patient and ends with the administration of the final product to the patient. Between collection and final administration of a product, there is a complex manufacturing process. This complexity brings unique supply chain challenges and need for enhanced visibility of the entire cell therapy process. This session will address the need for heightened chain of custody and identity though use of electronic record keeping for every step of the process, prescriptive workflows and standardized operating procedures, and a robust distribution that may include digital real-time tracking and improved containers to maximize product effectiveness.

Moderator: Lori Daane, Director of Scientific Affairs, *bioMérieux*

1:30 p.m. | Low-Temperature Storage and Preservation of Gene- and Cell-Based Drug Products
Matthew R. Gehrmann, Senior Scientist, *West Pharmaceutical Services, Inc.*

2:00 p.m. | Is Cell Therapy Manufacturing Fundamentally All about Supply Chain Challenges?
Patricia M. Seymour, MBA, CSCP, Managing Director, *BPTG of BDO USA*

2:30 p.m. | Questions and Answers/Discussion

3:00 p.m. – 3:45 p.m. | Refreshment Break and Poster Presentations in Exhibit Area

3:45 p.m. – 5:30 p.m. | P8: Navigating the Regulatory Pathway for Cell and Gene Therapy

In this session, we will have perspectives from regulators and industry on challenges and opportunities moving innovative cell and gene therapy products through the regulatory lifecycle towards commercialization. We will explore how cell and gene therapies are viewed in comparison to more traditional biologics and what the future looks like to ensure the industry can drive to meet patient needs in a safe, effective, and efficient process.

Moderator: David Smith, PhD, Associate Director, *Hitachi Chemical Advanced Therapeutics Solutions*

3:45 p.m. | Panel Discussion
EJ Brandreth, III, MBA, Senior Vice President, Quality, *Inovio Pharmaceuticals*
Arifa S. Khan, PhD, Supervisory Microbiologist, CBER, *FDA*
Dr. Mike Long, MBB, Senior Director Consulting Services, *ValSource LLC*
Steven S. Oh, PhD, Deputy Director, Division of Cellular and Gene Therapies, CBER, *FDA*

5:15 p.m. | Closing Remarks from Conference Co-Chair
Michael Blackton, MBA, Vice President, Global Quality, *Adaptimmune, LLC*